

What is claimed is:

- 5 1. A single cDNA comprising a nucleic acid sequence coding for cystic fibrosis
transmembrane conductance regulator.
- 10 2. The cDNA of claim 1 further comprising phage, viral, liposome or virosome
elements for enabling introduction of the cDNA encoding cystic fibrosis
transmembrane conductance regulator into a cell.
- 15 3. Therapeutically effective composition comprising the cDNA of claim 2 and a
carrier..
- 20 4. A vector comprising DNA encoding cystic fibrosis transmembrane conductance
regulator which, when therapeutically administered to patients suffering from
cystic fibrosis results in improved cystic fibrosis transmembrane conductance
regulator function.
- 25 5. The vector of claim 4 wherein the vector is present in a copy number which,
when the vector is therapeutically introduced into a human cell phenotypically
exhibiting characteristics of cystic fibrosis does not result in the production of
cystic fibrosis transmembrane conductance regulator in a quantity or
concentration which causes the host cell to die.
- 30 6. A phage, virus, liposome or virosome comprising the vector of claim 4.
7. A therapeutic composition capable of effecting the production, glycosylation
and transportation to the plasma membrane of cystic fibrosis transmembrane
conductance regulator.
8. The therapeutic composition of claim 7 which comprises a phage, virus, liposome
or virosome.

9. The therapeutic composition of claim 8 which further comprises the vector of claim 4.

5 10. A therapeutic composition comprising a carrier comprising the cDNA of claim 1 which after administration, augments the in vivo production or activity of atleast partially glycosylated cystic fibrosis transmembrane conductance regulator in the plasma membrane of human cells without overloading transport mechanisms to and from endoplasmic reticulum or Golgi apparatus of such cells.

10 11. A method for diagnosing cystic fibrosis transmembrane conductance regulator dysfunction in mammalian host cells comprising the step of identifying the presence or absence of band C of cystic fibrosis transmembrane conductance regulator isolated from such cells.

15 12. The method of claim 11 which further comprises identifying the amount of non-glycosylated and partially glycosylated cystic fibrosis transmembrane conductance regulator associated with said cell and correlating said amounts with cystic fibrosis genetic mutations.

20 13. A method for treating a disease condition having the characteristics of cystic fibrosis comprising the step of administering to cells having defective cystic fibrosis transmembrane conductance regulator function a therapeutically effective dose of the cDNA of claim 1 wherein such cDNA results in expression of cystic fibrosis transmembrane conductance regulator in an amount which does not overload the cystic fibrosis transmembrane conductance regulator associated transport mechanisms in such cells.

14. A method of the cDNA of claim 1 wherein such cDNA results in expression of cystic fibrosis transmembrane conductance regulator in an amount which does not overload the cystic fibrosis transmembrane conductance regulator associated transport mechanisms in such cells cystic fibrosis transmembrane conductance regulator.

15. The method of claim 14 which comprises administering said cystic fibrosis transmembrane conductance regulator in a pharmaceutically acceptable carrier by aerosol inhalation.

16. The method of claim 15 wherein nucleotide binding domain 2 of the cystic fibrosis transmembrane conductance regulator has been substituted for nucleotide binding domain 1.

17. A method for reducing cystic fibrosis transmembrane conductance regulator dysfunction resulting from excessive presence or activity thereof in non-plasma membrane locations in cystic fibrosis cells comprising administering an effective amount of an agent for deactivating the non-plasma membrane located cystic fibrosis transmembrane conductance regulator or causing the transport of said cystic fibrosis transmembrane conductance regulator to the plasma membrane.

18. The method of claim 17 wherein said agent results in the addition of N-linked carbohydrate to the cystic fibrosis transmembrane conductance regulator.

19. The method of claim 17 wherein said agent simulates the nucleotide binding domain activity of the cystic fibrosis transmembrane conductance regulator at the endoplasmic reticulum of said cystic fibrosis cells thereby causing glycosylation of the cystic fibrosis transmembrane conductance regulator to occur.

20. The method to claim 13 wherein said cDNA homologously combines with the cystic fibrosis gene of said cell such that resultant protein contains the correct wild-type amino acid sequence of human cystic fibrosis transmembrane conductance regulator.
21. The method of claim 20 wherein said cells are from a CF patient exhibiting a ΔF 508 mutation and following said administering step, said resultant protein contains phenylalanine at position 508.
22. A method for producing antibodies specific for cystic fibrosis transmembrane conductance regulator comprising the step of forming a fusion protein comprising a first protein and a polypeptide comprising at least one cystic fibrosis transmembrane conductance regulator domain, employing said fusion protein as an immunogen and collecting antibodies formed in response to said immunogen.
23. The antibody produced by the method of claim 22 which is specific for an epitope of the cystic fibrosis transmembrane conductance regulator.
24. The antibody of claim 23 wherein said epitope is associated with a region selected from Exon 1, Exon 10, Exon 24, extracellular loops region of approximately amino acids 139-194 and extracellular loop region of approximately amino acids 881-911.

add C² add 7³ add 8²

add H¹ add L¹